

10/054665  
Search results

## Refine Search

## Search Results -

Terms	Documents
L6 and splic\$ near5 transcript\$	6

Database:

US Pre-Grant Publication Full-Text Database  
 US Patents Full-Text Database  
 US OCR Full-Text Database  
 EPO Abstracts Database  
 JPO Abstracts Database  
 Derwent World Patents Index  
 IBM Technical Disclosure Bulletins

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L7

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## Search History

DATE: Saturday, June 05, 2004 [Printable Copy](#) [Create Case](#)

<u>Set</u> <u>Name</u> side by side	<u>Query</u>	<u>Hit</u> <u>Count</u>	<u>Set</u> <u>Name</u> result set
	DB=PGPB,USPT,USOC,EPAB,JPAB,DWPI,TDBD; PLUR=YES; OP=OR		
<u>L7</u>	L6 and splic\$ near5 transcript\$	6	<u>L7</u>
<u>L6</u>	first near5 (AAV or adeno near associated) and second near5 (AAV or adeno near associated) and splice near donor and splice near acceptor	17	<u>L6</u>
<u>L5</u>	L4 and trans near splic\$	18	<u>L5</u>
<u>L4</u>	L3 and splice near donor and splice near acceptor	260	<u>L4</u>
<u>L3</u>	(AAV\$ or adeno near associated near vir\$)	16050	<u>L3</u>
<u>L2</u>	L1 and enhancer\$	1	<u>L2</u>
<u>L1</u>	6436392 [pn]	2	<u>L1</u>

END OF SEARCH HISTORY

[Generate Collection](#)[Print](#)**Search Results - Record(s) 1 through 18 of 18 returned.**

- 
- ☐ 1. 20040058344. 25 Feb 03. 25 Mar 04. Trans-splicing mediated imaging of gene expression. Mitchell, Lloyd G., et al. 435/6; 435/320.1 435/325 435/455 435/69.1 C12Q001/68 C12P021/02 C12N005/06 C12N015/85.
- 
- ☐ 2. 20040038396. 05 Jun 02. 26 Feb 04. Spliceosome mediated RNA trans-splicing for correction of factor VIII genetic defects. Mitchell, Lloyd G., et al. 435/325; C12N005/06.
- 
- ☐ 3. 20040018622. 17 Jul 02. 29 Jan 04. Spliceosome mediated RNA trans-splicing for correction of skin disorders. Mitchell, Lloyd G., et al. 435/371; 424/93.21 514/44 A61K048/00 C12N005/08.
- 
- ☐ 4. 20030204861. 30 Apr 02. 30 Oct 03. Transgenic animal model for spliceosome-mediated RNA trans-splicing. Puttaraju, Madaiah, et al. 800/18; A01K067/027.
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- ☐ 5. 20030153054. 12 Feb 02. 14 Aug 03. Methods and compositions for use in spliceosome mediated RNA trans-splicing. Mitchell, Lloyd G., et al. 435/91.2; 435/375 C12P019/34 C12N005/00.
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- ☐ 6. 20030148937. 20 Apr 01. 07 Aug 03. Methods and compositions for use in spliceosome mediated RNA trans-splicing. Mansfield, S. Gary, et al. 514/12; 435/325 435/6 530/383 A61K038/37 C12Q001/68 C12N005/06 C07K014/755.
- 
- ☐ 7. 20030103939. 12 Jul 02. 05 Jun 03. Pseudotyped adeno-associated viruses and uses thereof. Engelhardt, John F., et al. 424/93.2; 435/235.1 435/320.1 435/456 514/44 A61K048/00 C12N007/00 C12N015/861.
- 
- ☐ 8. 20030077754. 08 Jan 01. 24 Apr 03. Methods and compositions for use in spliceosome mediated RNA trans-splicing. Mitchell, Lloyd G., et al. 435/69.7; 435/183 435/320.1 435/325 536/23.2 C12N009/00 C07H021/04 C12P021/04 C12N005/06.
- 
- ☐ 9. 20030027250. 29 Aug 01. 06 Feb 03. Methods and compositions for use in spliceosome mediated RNA trans-splicing. Mitchell, Lloyd G., et al. 435/69.1; 435/325 435/456 C12P021/02 C12N005/06 C12N015/86.
- 
- ☐ 10. 20020197237. 22 Jan 02. 26 Dec 02. Adeno-associated virus vectors. Engelhardt, John F., et al. 424/93.2; 435/235.1 435/320.1 435/456 A61K048/00 C12N015/861 C12N007/00.
- 
- ☐ 11. 20020193580. 12 Feb 02. 19 Dec 02. Methods and compositions for use in spliceosome mediated RNA trans-splicing. Mitchell, Lloyd G., et al. 536/23.1; C07H021/02 C07H021/04.
- 
- ☐ 12. 20020115207. 08 Jan 01. 22 Aug 02. Methods and compositions for use in spliceosome mediated RNA trans-splicing. Mitchell, Lloyd G., et al. 435/325; 435/320.1 536/23.1 C12P021/02 C12N005/06 C07H021/04.
- 
- ☐ 13. 20020064523. 23 Dec 98. 30 May 02. SYNTHETIC MULTIVULVA (SYNMUV) POLYPEPTIDES. HORVITZ, H ROBERT, et al. 424/94.1; A61K038/43.
-

☐ 14. 6436392. 25 Mar 99; 20 Aug 02. Adeno-associated virus vectors. Engelhardt; John F., et al. 424/93.2; 424/93.1 424/93.6 435/320.1 435/455 435/456 435/457 435/69.1 435/91.4 435/91.41. A61K048/00 C12N015/864 C12N015/63 C12N015/64.

☐ 15. 6280978. 23 Sep 98; 28 Aug 01. Methods and compositions for use in spliceosome mediated RNA trans-splicing. Mitchell; Lloyd G., et al. 435/91.3; 435/320.1 435/325 435/455 435/91.31 536/23.1. C07H021/02 C12N005/10 C12N015/09 C12P019/34.

☐ 16. 6083702. 13 Aug 98; 04 Jul 00. Methods and compositions for use in spliceosome mediated RNA trans-splicing. Mitchell; Lloyd G., et al. 435/6; 435/320.1 435/325 435/455 435/91.1 514/44 536/23.1. A61K048/00 C12N005/10 C12P019/34 C12Q001/68.

☐ 17. 6015709. 27 Aug 97; 18 Jan 00. Transcriptional activators, and compositions and uses related thereto. Natesan; Sridaran. 435/366; 435/252.3 435/254.11 435/325 536/23.4. C12N001/15 C12N001/21 C12N005/10 C12N015/62.

☐ 18. US 6436392B. Recombinant adeno-associated virus vector useful for gene therapy against disorders related to blood, neurological and muscular systems. DUAN, D, et al. A01K067/027 A61K031/70 A61K031/711 A61K048/00 C07K014/47 C12N005/10 C12N007/00 C12N015/00 C12N015/10 C12N015/63 C12N015/64 C12N015/86 C12N015/861 C12N015/864.

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Terms	Documents
L4 and trans near splic\$	18

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Day : Saturday  
Date: 6/5/2004  
Time: 13:46:47

## Inventor Name Search

Enter the **first few letters** of the Inventor's Last Name.  
Additionally, enter the **first few letters** of the Inventor's First name.

**Last Name**

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Day : Saturday  
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**Last Name**

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Set	Items	Description
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? set hi ;set hi		
HILIGHT set on as ''		
HILIGHT set on as ''		
? begin 5,6,154,155,156,312,399,biotech,biosci		
>>>	135 is unauthorized	

Set	Items	Description
?	s	(AAV? or adeno (n) associated) and splice (n) donor? and splice (n) acceptor?
Processed 10 of 33 files ...		
Processing		
Completed processing all files		
	14244	AAV?
	53020	ADENO
	6817012	ASSOCIATED
	19322	ADENO(N)ASSOCIATED
	95369	SPLICE
	903506	DONOR?
	10345	SPLICE(N)DONOR?
	95369	SPLICE
	251778	ACCEPTOR?
	7180	SPLICE(N)ACCEPTOR?
S1	26	(AAV? OR ADENO (N) ASSOCIATED) AND SPLICE (N) DONOR? AND SPLICE (N) ACCEPTOR?
? rd s1		
...completed examining records		
S2	15	RD S1 (unique items)
? s s2 and trans (n) splic?		
	15	S2
	698965	TRANS
	263027	SPLIC?
	5039	TRANS(N)SPLIC?
S3	1	S2 AND TRANS (N) SPLIC?
? s s3/9/1		
>>>Invalid syntax		
? d s3/9/1		
Display 3/9/1 (Item 1 from file: 5)		
DIALOG(R)File 5:Biosis Previews(R)		
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0014206953 BIOSIS NO.: 200300165672

**Trans-Splicing AAV** Vector System Expands the Packaging

Capacity Of **AAV** Gene Therapy Vectors for Delivery of Large Transgenes to the Retina.

AUTHOR: Reich S J (Reprint); Maguire A M (Reprint); Auricchio A; Hildinger M; Wilson J M; Bennett J (Reprint)

AUTHOR ADDRESS: Dept. of Ophthalmology, Scheie Eye Inst., University of Pennsylvania, Philadelphia, PA, USA\*\*USA

JOURNAL: ARVO Annual Meeting Abstract Search and Program Planner 2002 p Abstract No. 4589 2002 2002

MEDIUM: cd-rom

CONFERENCE/MEETING: Annual Meeting of the Association For Research in Vision and Ophthalmology Fort Lauderdale, Florida, USA May 05-10, 2002; 20020505

DOCUMENT TYPE: Meeting; Meeting Abstract

RECORD TYPE: Abstract

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DIALOG(R)File 5:Biosis Previews(R)  
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LANGUAGE: English

ABSTRACT: Purpose: **Adeno-Associated Virus (AAV)** based

recombinant vectors can safely transduce different retinal cell types resulting in efficient gene expression. A major shortcoming of this vector is its small packaging capacity that limits the delivery of large

transgene cassettes. A **\*\*\*trans\*\*\* - \*\*\*splicing\*\*\*** approach has been developed, which reconstitutes gene expression from two independent **AAV** vectors, in order to overcome the vector's packaging restrictions. Our aim was to determine whether this **\*\*\*trans\*\*\* - \*\*\*splicing\*\*\*** **\*\*\*AAV\*\*\*** system would work in the context of the retina. Methods: **\*\*\*AAV2\*\*\*** .1lacZ-SD contains the upstream half of the lacZ cDNA followed by a **splice donor** site under the control of the cytomegalovirus (CMV) promoter. **\*\*\*AAV2\*\*\*** .1lacZ-SA contains the downstream half of the lacZ cDNA initiated by a **splice \*\*\*acceptor\*\*\*** site. Both vectors were mixed at a 1:1 ratio and delivered unilaterally to eyes of CD1 mice by subretinal or intravitreal injection.

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The contralateral eyes received **AAV** carrying the gene encoding the intact lacZ gene as control. **\*\*\*AAV2\*\*\*** .1lacZ-SD and **\*\*\*AAV2\*\*\*** .1lacZ-SA were also delivered individually to eyes of additional CD1 mice to show there is no gene expression when the vectors are used alone. At 2-6 weeks post-injection, the mice were sacrificed and the effects of the treatments were evaluated by histochemical assay. Results: By bright field microscopy, lacZ expression was evident as early as 2 weeks and the level of expression increased with time up to 4 weeks. Expression persisted through the latest timepoint (6 weeks). All retinal cells known to be targeted by **AAV2** were positive for transgene expression, including retinal pigment epithelium cells, photoreceptors and ganglion cells. Eyes from mice treated with either **\*\*\*AAV2\*\*\*** .1lacZ-SD or **\*\*\*AAV2\*\*\*** .1lacZ-SA alone showed no lacZ expression. Eyes from mice treated with the **\*\*\*AAV\*\*\*** .lacZ control vector were similar except that lacZ expression was significantly stronger and more widespread than in experimentals at every timepoint. Conclusion: The **\*\*\*trans\*\*\* - **splicing AAV**** vector system can transduce cells of the retina

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when delivered by sub-retinal injection. This supports the potential utility of **trans-splicing AAV** as a vector for the treatment of diseases of the retina that require delivery of genes that exceed the packaging limits of conventional **\*\*\*AAV\*\*\*** vectors.

#### DESCRIPTORS:

MAJOR CONCEPTS: Molecular Genetics--Biochemistry and Molecular Biophysics  
; Sense Organs--Sensory Reception; Vector Biology  
BIOSYSTEMATIC NAMES: Parvoviridae--ssDNA Viruses, Viruses, Microorganisms  
; Muridae--Rodentia, Mammalia, Vertebrata, Chordata, Animalia  
ORGANISMS: **adeno-associated virus {AAV}** (Parvoviridae)  
--gene vector; mouse (Muridae)  
ORGANISMS: PARTS ETC: retina--sensory system; eye--sensory system  
COMMON TAXONOMIC TERMS: Single-Stranded DNA Viruses; Microorganisms;  
Viruses; Animals; Chordates; Mammals; Nonhuman Vertebrates; Nonhuman  
Mammals; Rodents; Vertebrates  
CHEMICALS & BIOCHEMICALS: transgenes

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METHODS & EQUIPMENT: **Adeno-Associated** Virus gene therapy--  
clinical techniques, therapeutic and prophylactic techniques;  
**trans-splicing adeno-associated** virus vector  
system--laboratory equipment

MISCELLANEOUS TERMS: packaging capacity; Meeting Abstract

CONCEPT CODES:

00520 General biology - Symposia, transactions and proceedings  
03502 Genetics - General  
03506 Genetics - Animal  
20004 Sense organs - Physiology and biochemistry  
31500 Genetics of bacteria and viruses  
33502 Virology - General and methods  
37057 Public health: disease vectors - General

BIOSYSTEMATIC CODES:

03205 Parvoviridae  
86375 Muridae

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0014206953 BIOSIS NO.: 200300165672

Trans-Splicing **AAV** Vector System Expands the Packaging Capacity Of  
**AAV** Gene Therapy Vectors for Delivery of Large Transgenes to the  
Retina.

AUTHOR: Reich S J (Reprint); Maguire A M (Reprint); Auricchio A; Hildinger  
M; Wilson J M; Bennett J (Reprint)

AUTHOR ADDRESS: Dept. of Ophthalmology, Scheie Eye Inst., University of  
Pennsylvania, Philadelphia, PA, USA\*\*USA

JOURNAL: ARVO Annual Meeting Abstract Search and Program Planner 2002 p  
Abstract No. 4589 2002 2002

MEDIUM: cd-rom

CONFERENCE/MEETING: Annual Meeting of the Association For Research in  
Vision and Ophthalmology Fort Lauderdale, Florida, USA May 05-10, 2002;  
20020505

DOCUMENT TYPE: Meeting; Meeting Abstract

RECORD TYPE: Abstract

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LANGUAGE: English

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Display 2/3/2 (Item 2 from file: 5)

DIALOG(R)File 5:Biosis Previews(R)

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0013150161 BIOSIS NO.: 200100322000

Novel method for human factor VIII packaging and expression: Dimerization  
of rAAV vectors

AUTHOR: Chao Hengjun (Reprint); Walsh Christopher E (Reprint)

AUTHOR ADDRESS: UNC Gene Therapy Center, University of North Carolina at  
Chapel Hill, Chapel Hill, NC, USA\*\*USA

JOURNAL: Blood 96 (11 Part 1): p216a-217a November 16, 2000 2000

MEDIUM: print

CONFERENCE/MEETING: 42nd Annual Meeting of the American Society of

Hematology San Francisco, California, USA December 01-05, 2000; 20001201  
SPONSOR: American Society of Hematology  
ISSN: 0006-4971  
DOCUMENT TYPE: Meeting; Meeting Abstract  
RECORD TYPE: Abstract  
LANGUAGE: English

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Display 2/3/3 (Item 3 from file: 5)  
DIALOG(R)File 5:BIOSIS Previews(R)  
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0012628031 BIOSIS NO.: 200000346344  
Increasing the size of rAAV-mediated expression cassettes in vivo by  
intermolecular joining of two complementary vectors  
AUTHOR: Nakai Hiroyuki; Storm Theresa A; Kay Mark A (Reprint)  
AUTHOR ADDRESS: Program in Human Gene Therapy, Department of Pediatrics and  
Genetics, Stanford University, Stanford, CA, 94305, USA\*\*USA  
JOURNAL: Nature Biotechnology 18 (5): p527-532 May, 2000 2000  
MEDIUM: print  
ISSN: 1087-0156  
DOCUMENT TYPE: Article  
RECORD TYPE: Abstract  
LANGUAGE: English

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Display 2/3/4 (Item 1 from file: 154)  
DIALOG(R)File 154:MEDLINE(R)  
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11839506 PMID: 12027555  
Expression of human factor VIII by splicing between dimerized **AAV**  
vectors.  
Chao Hengjun; Sun Liangwu; Bruce Andrew; Xiao Xiao; Walsh Christopher E  
UNC Gene Therapy Center, University of North Carolina at Chapel Hill,  
North Carolina 27599, USA.  
Molecular therapy - the journal of the American Society of Gene Therapy (  
United States) Jun 2002, 5 (6) p716-22, ISSN 1525-0016  
Journal Code: 100890581  
Document type: Journal Article  
Languages: ENGLISH  
Main Citation Owner: NLM  
Record type: Completed

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Display 2/3/5 (Item 1 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
(c) 2004 American Chemical Society. All rts. reserv.

140123648 CA: 140(9)123648j PATENT  
Compositions and methods for making mutations in cell lines and animals  
by physicochem. treatment and insertional gene trap vectors  
INVENTOR(AUTHOR): Harrington, John Joseph; Jackson, Paul David; Jiang, Li  
LOCATION: USA  
ASSIGNEE: Athersys, Inc.  
PATENT: U.S. Pat. Appl. Publ. ; US 20040018624 A1 DATE: 20040129  
APPLICATION: US 277612 (20021022) \*US PV336497 (20011022) \*US 196721  
(20020715)  
PAGES: 59 pp., Cont.-in-part of U.S. Ser. No. 196,721, abandoned.  
CODEN: USXXCO LANGUAGE: English CLASS: 435455000; C12N-015/85A

- end of record -

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Display 2/3/6 (Item 2 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
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140019765 CA: 140(2)19765j PATENT  
Compns. and methods for making and detecting gene mutations in transgenic  
cell lines and animals  
INVENTOR(AUTHOR): Harrington, John Joseph; Jackson, Paul David; Jiang, Li  
LOCATION: USA  
ASSIGNEE: Athersys, Inc.  
PATENT: U.S. Pat. Appl. Publ. ; US 20030224519 A1 DATE: 20031204  
APPLICATION: US 345115 (20030115) \*US PV336497 (20011022) \*US 196721  
(20020715) \*US 277612 (20021022)  
PAGES: 61 pp., Cont.-in-part of U.S. Ser. No. 277,612. CODEN: USXXCO  
LANGUAGE: English CLASS: 435441000; C12Q-001/68A; C12N-015/01B

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DIALOG(R)File 399:CA SEARCH(R)  
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139241300 CA: 139(16)241300s PATENT  
Helper constructs for the propagation of hybrid adeno-associated virus  
particles using genes from different virus serotypes  
INVENTOR(AUTHOR): Hoerer, Markus; Dubielzig, Ralf; Ries, Stefan;  
Kosfeld-Bergauer, Meike  
LOCATION: Germany,  
ASSIGNEE: Medigene A.-G.  
PATENT: PCT International ; WO 200374686 A1 DATE: 20030912  
APPLICATION: WO 2003EP2351 (20030307) \*DE 10210139 (20020307)  
PAGES: 43 pp. CODEN: PIXXD2 LANGUAGE: German CLASS: C12N-005/10A;  
C12N-015/86B; C12N-015/00B DESIGNATED COUNTRIES: AE; AG; AL; AM; AT; AU;  
AZ; BA; BB; BG; BR; BY; BZ; CA; CH; CN; CO; CR; CU; CZ; DE; DK; DM; DZ; EC;  
EE; ES; FI; GB; GD; GE; GH; GM; HR; HU; ID; IL; IN; IS; JP; KE; KG; KP; KR;  
KZ; LC; LK; LR; LS; LT; LU; LV; MA; MD; MG; MK; MN; MW; MX; MZ; NO; NZ; OM;  
PH; PL; PT; RO; RU; SC; SD; SE; SG; SK; SL; TJ; TM; TN; TR; TT; TZ; UA; UG;  
US; UZ; VC; VN; YU; ZA; ZM; ZW; AM; AZ; BY; KG; KZ; MD; RU; TJ; TM

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DESIGNATED REGIONAL: GH; GM; KE; LS; MW; MZ; SD; SL; SZ; TZ; UG; ZM; ZW;  
AT; BE; BG; CH; CY; CZ; DE; DK; EE; ES; FI; FR; GB; GR; HU; IE; IT; LU; MC;  
NL; PT; RO; SE; SI; SK; TR; BF; BJ; CF; CG; CI; CM; GA; GN; GQ; GW; ML; MR;  
NE; SN; TD; TG

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Display 2/3/8 (Item 4 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
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138332879 CA: 138(22)332879h PATENT  
Insertional and physicochemical mutagenesis methods for making homozygous  
gene mutations in cell lines and animals  
INVENTOR(AUTHOR): Harrington, John Joseph; Jackson, Paul David; Jiang, Li  
LOCATION: USA

ASSIGNEE: Athersys, Inc.  
PATENT: PCT International ; WO 200335834 A2 DATE: 20030501  
APPLICATION: WO 2002US33714 (20021022) \*US PV336497 (20011022) \*US 196721  
(20020715)

PAGES: 165 pp. CODEN: PIXXD2 LANGUAGE: English CLASS: C12N-000/A  
DESIGNATED COUNTRIES: AE; AG; AL; AM; AT; AU; AZ; BA; BB; BG; BR; BY; BZ;  
CA; CH; CN; CO; CR; CU; CZ; DE; DK; DM; DZ; EC; EE; ES; FI; GB; GD; GE; GH;  
GM; HR; HU; ID; IL; IN; IS; JP; KE; KG; KP; KR; KZ; LC; LK; LR; LS; LT; LU;  
LV; MA; MD; MG; MK; MN; MW; MX; MZ; NO; NZ; OM; PH; PL; PT; RO; RU; SD; SE;  
SG; SI; SK; SL; TJ; TM; TN; TR; TT; TZ; UA; UG; US; UZ; VC; VN; YU; ZA; ZM;  
ZW; AM; AZ; BY; KG; KZ; MD; RU; TJ; TM DESIGNATED REGIONAL: GH; GM; KE; LS

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; MW; MZ; SD; SL; SZ; TZ; UG; ZM; ZW; AT; BE; BG; CH; CY; CZ; DE; DK; EE;  
ES; FI; FR; GB; GR; IE; IT; LU; MC; NL; PT; SE; SK; TR; BF; BJ; CF; CG; CI;  
CM; GA; GN; GQ; GW; ML; MR; NE; SN; TD; TG

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Display 2/3/9 (Item 5 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
(c) 2004 American Chemical Society. All rts. reserv.

127268014 CA: 127(19)268014r PATENT  
Antisense approach to gene therapy of viral diseases  
INVENTOR(AUTHOR): Feldman, Lawrence T.  
LOCATION: USA  
ASSIGNEE: Regents of the University of California  
PATENT: PCT International ; WO 9734638 A1 DATE: 19970925  
APPLICATION: WO 97US4519 (19970320) \*US 13796 (19960320)  
PAGES: 32 pp. CODEN: PIXXD2 LANGUAGE: English CLASS: A61K-048/00A;  
C07H-021/04B; C12N-015/63B; C12N-015/85B DESIGNATED COUNTRIES: AU; CA; JP  
DESIGNATED REGIONAL: AT; BE; CH; DE; DK; ES; FI; FR; GB; GR; IE; IT; LU;  
MC; NL; PT; SE

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Display 2/3/10 (Item 1 from file: 98)  
DIALOG(R)File 98:General Sci Abs/Full-Text  
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03253333 H.W. WILSON RECORD NUMBER: BGSI96003333 (USE FORMAT 7 FOR  
FULLTEXT)  
Cystic fibrosis: genotypic and phenotypic variations.  
Zielenski, Julian  
Tsui, Lap-Chee  
Annual Review of Genetics (Annu Rev Genet) v. 29 ('95) p. 777-807  
SPECIAL FEATURES: bibl il ISSN: 0066-4197  
LANGUAGE: English  
COUNTRY OF PUBLICATION: United States  
WORD COUNT: 14040

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DIALOG(R)File 98:General Sci Abs/Full-Text  
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03051107 H.W. WILSON RECORD NUMBER: BGSI95051107 (USE FORMAT 7 FOR

FULLTEXT)

Viral vectors in gene therapy.

Smith, Alan E

Annual Review of Microbiology (Annu Rev Microbiol) v. 49 ('95) p. 807-38

DOCUMENT TYPE: Feature Article

SPECIAL FEATURES: bibl il ISSN: 0066-4227

LANGUAGE: English

COUNTRY OF PUBLICATION: United States

WORD COUNT: 14692

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Display 2/3/12 (Item 1 from file: 357)

DIALOG(R)File 357:Derwent Biotech Res.

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0333782 DBR Accession No.: 2004-06074 PATENT

New vector comprising a polynucleotide encoding a calbindin-D28k polypeptide, useful for treating glucocorticoid induced cell death, particularly glucocorticoid-induced osteoporosis - recombinant virus vector expression in host cell for use in disease gene therapy

AUTHOR: CHRISTAKOS S

PATENT ASSIGNEE: UNIV NEW JERSEY MEDICINE and DENTISTRY 2003

PATENT NUMBER: US 20030219421 PATENT DATE: 20031127 WPI ACCESSION NO.: 2004-021929 (200402)

PRIORITY APPLIC. NO.: US 155567 APPLIC. DATE: 20020523

NATIONAL APPLIC. NO.: US 155567 APPLIC. DATE: 20020523

LANGUAGE: English

- end of record -

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Display 2/3/13 (Item 2 from file: 357)

DIALOG(R)File 357:Derwent Biotech Res.

(c) 2004 Thomson Derwent & ISI. All rts. reserv.

0309214 DBR Accession No.: 2003-10999 PATENT

Altering recombinant **adeno-associated** virus (rAAV) transduction of mammalian cell by contacting cell with pseudotyped rAAV having combination of **AAV** capsid protein and rAAV genome, and agent that alters virus transduction - recombinant **adeno-associated** virus vector-mediated gene transfer and expression in host cell for use in gene therapy

AUTHOR: ENGELHARDT J F; YAN Z

PATENT ASSIGNEE: UNIV IOWA RES FOUND; ENGELHARDT J F; YAN Z 2003

PATENT NUMBER: WO 2003006616 PATENT DATE: 20030123 WPI ACCESSION NO.: 2003-229480 (200322)

PRIORITY APPLIC. NO.: US 305204 APPLIC. DATE: 20010713

NATIONAL APPLIC. NO.: WO 2002US21926 APPLIC. DATE: 20020712

LANGUAGE: English

- end of record -

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Display 2/3/14 (Item 3 from file: 357)

DIALOG(R)File 357:Derwent Biotech Res.

(c) 2004 Thomson Derwent & ISI. All rts. reserv.

0291802 DBR Accession No.: 2002-13649 PATENT

Identifying active genomic polynucleotides comprises contacting living cells with substrate for product of reporter gene which was integrated into genome by **adeno-associated** viral vector, and sorting cells by fluorescence - drug screening involving **adeno-associated** virus vector-mediated reporter gene expression in eukaryote cell

AUTHOR: WHITNEY M A; XANTHOPOULOS K; NELSON D; NEGULESCU P; CRAIG F;  
FOULKES J G  
PATENT ASSIGNEE: AURORA BIOSCIENCES CORP 2002  
PATENT NUMBER: US 20020025940 PATENT DATE: 20020228 WPI ACCESSION NO.:  
2002-255974 (200230)  
PRIORITY APPLIC. NO.: US 772114 APPLIC. DATE: 20010126  
NATIONAL APPLIC. NO.: US 772114 APPLIC. DATE: 20010126  
LANGUAGE: English

- end of record -

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Display 2/3/15 (Item 4 from file: 357)  
DIALOG(R)File 357:Derwent Biotech Res.  
(c) 2004 Thomson Derwent & ISI. All rts. reserv.

0217693 DBR Accession No.: 97-12814 PATENT  
Producing a splicing cassette enabling production of stable antisense  
introns - retro virus, adeno virus and **adeno-associated**  
virus vector-mediated antisense expression in e.g. SUPT1, CD34, CD4 and  
hematopoietic stem and progenitor cell for gene therapy  
AUTHOR: Feldman L T  
CORPORATE SOURCE: Oakland, CA, USA.  
PATENT ASSIGNEE: Univ.California 1997  
PATENT NUMBER: WO 9734638 PATENT DATE: 970925 WPI ACCESSION NO.:  
97-479999 (9744)  
PRIORITY APPLIC. NO.: US 13796 APPLIC. DATE: 960320  
NATIONAL APPLIC. NO.: WO 974519 APPLIC. DATE: 970320  
LANGUAGE: English

- end of record -

?

? s (AAV? or adeno (n) associated) and trans (n) splic?  
14244 AAV?  
53020 ADENO  
6817012 ASSOCIATED  
19322 ADENO(N)ASSOCIATED  
698965 TRANS  
263027 SPLIC?  
5039 TRANS(N)SPLIC?  
S4 70 (AAV? OR ADENO (N) ASSOCIATED) AND TRANS (N) SPLIC?  
? rd s4  
...examined 50 records (50)  
...completed examining records  
S5 31 RD S4 (unique items)  
? d s5/3/1-31  
Display 5/3/1 (Item 1 from file: 5)  
DIALOG(R)File 5:Biosis Previews(R)  
(c) 2004 BIOSIS. All rts. reserv.

0014489526 BIOSIS NO.: 200300448205  
Double strand interaction is the predominant pathway for intermolecular  
recombination of \*\*\*adeno\*\*\* - \*\*\*associated\*\*\* viral genomes.  
AUTHOR: Yue Yongping; Duan Dongsheng (Reprint)  
AUTHOR ADDRESS: Department of Molecular Microbiology and Immunology,  
University of Missouri, School of Medicine, 1 Hospital Dr., Room M610G,  
MSB, Columbia, MO, 65212, USA\*\*USA  
AUTHOR E-MAIL ADDRESS: duand@health.missouri.edu  
JOURNAL: Virology 313 (1): p1-7 August 15, 2003 2003  
MEDIUM: print  
ISSN: 0042-6822 (ISSN print)  
DOCUMENT TYPE: Article  
RECORD TYPE: Abstract  
LANGUAGE: English

- end of record -

?

Display 5/3/2 (Item 2 from file: 5)  
DIALOG(R)File 5:Biosis Previews(R)  
(c) 2004 BIOSIS. All rts. reserv.

0014267360 BIOSIS NO.: 200300226079  
Method and vector for producing and transferring **trans-spliced**  
peptides  
AUTHOR: Xiao Xiao (Reprint); Liu Paul Xiangoin  
AUTHOR ADDRESS: Waxford, PA, USA\*\*USA  
JOURNAL: Official Gazette of the United States Patent and Trademark Office  
Patents 1269 (2): Apr. 8, 2003 2003  
MEDIUM: e-file  
PATENT NUMBER: US 6544786 PATENT DATE GRANTED: April 08, 2003 20030408  
PATENT CLASSIFICATION: 435-325 PATENT ASSIGNEE: University of Pittsburgh  
of the Commonwealth of Higher Education, Pittsburgh, PA, USA  
PATENT COUNTRY: USA  
ISSN: 0098-1133 (ISSN print)  
DOCUMENT TYPE: Patent  
RECORD TYPE: Abstract  
LANGUAGE: English

- end of record -

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Display 5/3/3 (Item 3 from file: 5)  
DIALOG(R)File 5:Biosis Previews(R)  
(c) 2004 BIOSIS. All rts. reserv.

0014266531 BIOSIS NO.: 200300225250  
Consequences of DNA-dependent protein kinase catalytic subunit deficiency  
on recombinant **adeno-associated** virus genome circularization  
and heterodimerization in muscle tissue.  
AUTHOR: Duan Dongsheng (Reprint); Yue Yongping; Engelhardt John F  
AUTHOR ADDRESS: Department of Molecular Microbiology and Immunology, School  
of Medicine, University of Missouri, 1 Hospital Dr., Room M610G, MSB,  
Columbia, MO, 65212, USA\*\*USA  
AUTHOR E-MAIL ADDRESS: duand@health.missouri.edu  
JOURNAL: Journal of Virology 77 (8): p4751-4759 April 2003 2003  
MEDIUM: print  
ISSN: 0022-538X (ISSN print)  
DOCUMENT TYPE: Article  
RECORD TYPE: Abstract  
LANGUAGE: English

- end of record -

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Display 5/3/4 (Item 4 from file: 5)  
DIALOG(R)File 5:Biosis Previews(R)  
(c) 2004 BIOSIS. All rts. reserv.

0014206953 BIOSIS NO.: 200300165672  
**Trans-Splicing AAV** Vector System Expands the Packaging  
Capacity Of **AAV** Gene Therapy Vectors for Delivery of Large  
Transgenes to the Retina.  
AUTHOR: Reich S J (Reprint); Maguire A M (Reprint); Auricchio A; Hildinger  
M; Wilson J M; Bennett J (Reprint)  
AUTHOR ADDRESS: Dept. of Ophthalmology, Scheie Eye Inst., University of  
Pennsylvania, Philadelphia, PA, USA\*\*USA  
JOURNAL: ARVO Annual Meeting Abstract Search and Program Planner 2002 p  
Abstract No. 4589 2002 2002  
MEDIUM: cd-rom

CONFERENCE/MEETING: Annual Meeting of the Association For Research in  
Vision and Ophthalmology Fort Lauderdale, Florida, USA May 05-10, 2002;  
20020505  
DOCUMENT TYPE: Meeting; Meeting Abstract  
RECORD TYPE: Abstract

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Display 5/3/4 (Item 4 from file: 5)  
DIALOG(R)File 5:Biosis Previews(R)  
(c) 2004 BIOSIS. All rts. reserv.  
LANGUAGE: English

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Display 5/3/5 (Item 5 from file: 5)  
DIALOG(R)File 5:Biosis Previews(R)  
(c) 2004 BIOSIS. All rts. reserv.

0014116584 BIOSIS NO.: 200300075303  
Efficient **trans-splicing** in the retina expands the utility of  
\*\*\*adeno\*\*\* - \*\*\*associated\*\*\* virus as a vector for gene therapy.  
AUTHOR: Reich S J; Auricchio A; Hildinger M; Glover E; Maguire A M; Wilson  
J M; Bennett J (Reprint)  
AUTHOR ADDRESS: F.M. Kirby Center for Molecular Ophthalmology, Scheie Eye  
Institute, 422 Curie Boulevard, 310 Stellar Chance Laboratories,  
Philadelphia, PA, 19104-6069, USA\*\*USA  
AUTHOR E-MAIL ADDRESS: jebennet@mail.med.upenn.edu  
JOURNAL: Human Gene Therapy 14 (1): p37-44 January 1, 2003 2003  
MEDIUM: print  
ISSN: 1043-0342 (ISSN print)  
DOCUMENT TYPE: Article  
RECORD TYPE: Abstract  
LANGUAGE: English

- end of record -

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Display 5/3/6 (Item 6 from file: 5)  
DIALOG(R)File 5:Biosis Previews(R)  
(c) 2004 BIOSIS. All rts. reserv.

0012630924 BIOSIS NO.: 200000349237  
**Trans-splicing** vectors expand the utility of **adeno-**  
**associated** virus for gene therapy  
AUTHOR: Yan Ziyang; Zhang Yulong; Duan Dongsheng; Engelhardt John F  
(Reprint)  
AUTHOR ADDRESS: Department of Anatomy and Cell Biology, University of Iowa  
College of Medicine, 51 Newton Road, Room 1-111 BSB, Iowa City, IA,  
52242, USA\*\*USA  
JOURNAL: Proceedings of the National Academy of Sciences of the United  
States of America 97 (12): p6716-6721 June 6, 2000 2000  
MEDIUM: print  
ISSN: 0027-8424  
DOCUMENT TYPE: Article  
RECORD TYPE: Abstract  
LANGUAGE: English

- end of record -

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Display 5/3/7 (Item 1 from file: 154)  
DIALOG(R)File 154:MEDLINE(R)  
(c) format only 2004 The Dialog Corp. All rts. reserv.



12189755 PMID: 12526170

**Trans-splicing** vectors expand the packaging limits of  
\*\*\*adeno\*\*\* - \*\*\*associated\*\*\* virus for gene therapy applications.  
Duan Dongsheng; Yue Yongping; Yan Ziyang; Engelhardt John F  
Department of Molecular Microbiology and Immunology, School of Medicine,  
University of Missouri, Columbia, MO, USA.  
Methods in molecular medicine (United States) 2003, 76 p287-307,  
ISSN 1543-1894 Journal Code: 101123138  
Contract/Grant No.: 2R01 HL 58340; HL; NHLBI; P30 DK54759; DK; NIDDK  
Document type: Journal Article  
Languages: ENGLISH  
Main Citation Owner: NLM  
Record type: Completed

- end of record -

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Display 5/3/8 (Item 2 from file: 154)  
DIALOG(R)File 154:MEDLINE(R)  
(c) format only 2004 The Dialog Corp. All rts. reserv.

11483388 PMID: 11592843

Expanding **AAV** packaging capacity with **trans-splicing** or  
overlapping vectors: a quantitative comparison.  
Duan D; Yue Y; Engelhardt J F  
Department of Anatomy & Cell Biology, Center for Gene Therapy of Cystic  
Fibrosis and Other Genetic Diseases, The University of Iowa, Iowa City,  
52242, USA. dongshen-duan@uiowa.edu  
Molecular therapy - the journal of the American Society of Gene Therapy (United States) Oct 2001, 4 (4) p383-91, ISSN 1525-0016  
Journal Code: 100890581  
Contract/Grant No.: HL58340; HL; NHLBI; P30 DK54759; DK; NIDDK  
Document type: Journal Article  
Languages: ENGLISH  
Main Citation Owner: NLM  
Record type: Completed

- end of record -

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Display 5/3/9 (Item 3 from file: 154)  
DIALOG(R)File 154:MEDLINE(R)  
(c) format only 2004 The Dialog Corp. All rts. reserv.

11137720 PMID: 11139798

Gene therapy: recombinant \*\*\*adeno\*\*\* - \*\*\*associated\*\*\* virus vectors.  
Smith-Arica J R; Bartlett J S  
Children's Research Institute, W531, 700 Children's Drive, Columbus, OH,  
43205-2696, USA. SmithJ@pediatrics.ohio-state.edu  
Current cardiology reports (United States) Jan 2001, 3 (1) p43-9,  
ISSN 1523-3782 Journal Code: 100888969  
Document type: Journal Article; Review; Review, Tutorial  
Languages: ENGLISH  
Main Citation Owner: NLM  
Record type: Completed

- end of record -

?

Display 5/3/10 (Item 1 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
(c) 2004 American Chemical Society. All rts. reserv.

138315154 CA: 138(21)315154a JOURNAL  
Hemophilia gene therapy: Novel rAAV vectors and RNA repair strategy  
AUTHOR(S): Chao, Hengjun; Walsh, Christopher E.

LOCATION: UNC Gene Therapy Center, Department of Medicine, University of North Carolina, Chapel Hill, NC, 27599, USA  
JOURNAL: Curr. Opin. Mol. Ther. (Current Opinion in Molecular Therapeutics) DATE: 2002 VOLUME: 4 NUMBER: 5 PAGES: 499-504 CODEN: CUOTFO ISSN: 1464-8431 LANGUAGE: English PUBLISHER: PharmaPress Ltd.

- end of record -

?

Display 5/3/11 (Item 2 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
(c) 2004 American Chemical Society. All rts. reserv.

138050493 CA: 138(5)50493b JOURNAL  
Recombinant AAV-mediated gene delivery using dual vector heterodimerization  
AUTHOR(S): Yan, Ziyang; Ritchie, Teresa C.; Duan, Dongsheng; Engelhardt, John F.  
LOCATION: Dep. Anatomy and Cell Biology, Univ. Iowa College Med., Iowa City, IA, 52242, USA  
JOURNAL: Methods Enzymol. (Methods in Enzymology) DATE: 2002 VOLUME: 346 NUMBER: Gene Therapy Methods PAGES: 334-357 CODEN: MENZAU ISSN: 0076-6879 LANGUAGE: English PUBLISHER: Academic Press

- end of record -

?

Display 5/3/12 (Item 3 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
(c) 2004 American Chemical Society. All rts. reserv.

137180771 CA: 137(13)180771f PATENT  
Adeno-associated virus trans-splicing vectors with increased episomal stability and gene therapy applications  
INVENTOR(AUTHOR): Engelhardt, John F.; Duan, Dongsheng  
LOCATION: USA  
ASSIGNEE: University of Iowa Research Foundation  
PATENT: United States ; US 6436392 B1 DATE: 20020820  
APPLICATION: US 276625 (19990325) \*US PV86166 (19980520)  
PAGES: 77 pp., Cont.-in-part of U.S. Provisional Ser. No. 86,166.  
CODEN: USXXAM LANGUAGE: English CLASS: 424093200; A61K-048/00A; C12N-015/864B; C12N-015/63B; C12N-015/64B

- end of record -

?

Display 5/3/13 (Item 4 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
(c) 2004 American Chemical Society. All rts. reserv.

136406835 CA: 136(26)406835y PATENT  
Procedure for the production of target-cell-specific pharmaceuticals  
LOCATION: Germany,  
ASSIGNEE: Dooley, Steven  
PATENT: Germany Offen. ; DE 10057014 A1 DATE: 20020529  
APPLICATION: DE 10057014 (20001117)  
PAGES: 18 pp. CODEN: GWXXBX LANGUAGE: German CLASS: A61K-048/00A; C12N-015/85B

- end of record -

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Display 5/3/14 (Item 5 from file: 399)  
DIALOG(R)File 399:CA SEARCH(R)  
(c) 2004 American Chemical Society. All rts. reserv.

134321588 CA: 134(23)321588d PATENT

Method and vector for producing and transferring trans-spliced peptides  
for the treatment of muscular dystrophy

INVENTOR(AUTHOR): Paul, X. Liu; Xiao, Xiao

LOCATION: Can.,

ASSIGNEE: Dalhousie University; University of Pittsburgh of the  
Commonwealth System of Higher Education

PATENT: PCT International ; WO 200129243 A1 DATE: 20010426

APPLICATION: WO 2000CA1216 (20001013) \*US PV159868 (19991015)

PAGES: 81 pp. CODEN: PIXXD2 LANGUAGE: English CLASS: C12N-015/864A;  
C12N-015/12B; C07K-014/47B; C12N-015/62B; C07K-019/00B; A61K-048/00B

DESIGNATED COUNTRIES: AE; AG; AL; AM; AT; AU; AZ; BA; BB; BG; BR; BY; BZ;  
CA; CH; CN; CR; CU; CZ; DE; DK; DM; DZ; EE; ES; FI; GB; GD; GE; GH; GM; HR;  
HU; ID; IL; IN; IS; JP; KE; KG; KP; KR; KZ; LC; LK; LR; LS; LT; LU; LV; MA;  
MD; MG; MK; MN; MW; MX; MZ; NO; NZ; PL; PT; RO; RU; SD; SE; SG; SI; SK; SL;  
TJ; TM; TR; TT; TZ; UA; UG; UZ; VN; YU; ZA; ZW; AM; AZ; BY; KG; KZ; MD; RU;

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Display 5/3/14 (Item 5 from file: 399)

DIALOG(R)File 399:CA SEARCH(R)

(c) 2004 American Chemical Society. All rts. reserv.

TJ; TM DESIGNATED REGIONAL: GH; GM; KE; LS; MW; MZ; SD; SL; SZ; TZ; UG; ZW  
; AT; BE; CH; CY; DE; DK; ES; FI; FR; GB; GR; IE; IT; LU; MC; NL; PT; SE;  
BF; BJ; CF; CG; CI; CM; GA; GN; GW; ML; MR; NE; SN; TD; TG

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Display 5/3/15 (Item 6 from file: 399)

DIALOG(R)File 399:CA SEARCH(R)

(c) 2004 American Chemical Society. All rts. reserv.

134306140 CA: 134(22)306140h PATENT

Adeno-associated viruses vectors utilizing splicing and gene therapy  
applications

INVENTOR(AUTHOR): Engelhardt, John F.; Dongsheng, Duan; Ziyang, Yan

LOCATION: USA

ASSIGNEE: University of Iowa Research Foundation

PATENT: PCT International ; WO 200125465 A1 DATE: 20010412

APPLICATION: WO 2000US27863 (20001006) \*US PV158209 (19991007)

PAGES: 144 pp. CODEN: PIXXD2 LANGUAGE: English CLASS: C12N-015/864A;  
C12N-007/01B; C12N-005/10B; A61K-048/00B DESIGNATED COUNTRIES: AE; AG; AL;  
AM; AT; AU; AZ; BA; BB; BG; BR; BY; BZ; CA; CH; CN; CR; CU; CZ; DE; DK; DM;  
DZ; EE; ES; FI; GB; GD; GE; GH; GM; HR; HU; ID; IL; IN; IS; JP; KE; KG; KP;  
KR; KZ; LC; LK; LR; LS; LT; LU; LV; MA; MD; MG; MK; MN; MW; MX; NO; NZ;  
PL; PT; RO; RU; SD; SE; SG; SI; SK; SL; TJ; TM; TR; TT; TZ; UA; UG; US; UZ;  
VN; YU; ZA; ZW; AM; AZ; BY; KG; KZ; MD; RU; TJ; TM DESIGNATED REGIONAL: GH  
; GM; KE; LS; MW; MZ; SD; SL; SZ; TZ; UG; ZW; AT; BE; CH; CY; DE; DK; ES;

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Display 5/3/15 (Item 6 from file: 399)

DIALOG(R)File 399:CA SEARCH(R)

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FI; FR; GB; GR; IE; IT; LU; MC; NL; PT; SE; BF; BJ; CF; CG; CI; CM; GA; GN;  
GW; ML; MR; NE; SN; TD; TG

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Display 5/3/16 (Item 7 from file: 399)

DIALOG(R)File 399:CA SEARCH(R)

(c) 2004 American Chemical Society. All rts. reserv.

132000950 CA: 132(1)950d PATENT

Adeno-associated virus vectors utilizing splicing and gene therapy applications in airway and muscle tissue

INVENTOR(AUTHOR): Engelhardt, John F.; Duan, Dongsheng

LOCATION: USA

ASSIGNEE: University of Iowa Research Foundation

PATENT: PCT International ; WO 9960146 A1 DATE: 19991125

APPLICATION: WO 99US11197 (19990520) \*US 86166 (19980520) \*US 276625 (19990325)

PAGES: 121 pp. CODEN: PIXXD2 LANGUAGE: English CLASS: C12N-015/86A; C12N-015/10B; C12N-005/10B; C07K-014/47B; A01K-067/027B; A61K-031/70B; A61K-048/00B DESIGNATED COUNTRIES: AE; AL; AM; AT; AU; AZ; BA; BB; BG; BR; BY; CA; CH; CN; CU; CZ; DE; DK; EE; ES; FI; GB; GD; GE; GH; GM; HR; HU; ID; IL; IN; IS; JP; KE; KG; KP; KR; KZ; LC; LK; LR; LS; LT; LU; LV; MD; MG; MK; MN; MW; MX; NO; NZ; PL; PT; RO; RU; SD; SE; SG; SI; SK; SL; TJ; TM; TR; TT; UA; UG; US; UZ; VN; YU; ZA; ZW; AM; AZ; BY; KG; KZ; MD; RU; TJ; TM

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Display 5/3/16 (Item 7 from file: 399)

DIALOG(R)File 399:CA SEARCH(R)

(c) 2004 American Chemical Society. All rts. reserv.

DESIGNATED REGIONAL: GH; GM; KE; LS; MW; SD; SL; SZ; UG; ZW; AT; BE; CH; CY; DE; DK; ES; FI; FR; GB; GR; IE; IT; LU; MC; NL; PT; SE; BF; BJ; CF; CG; CI; CM; GA; GN; GW; ML; MR; NE; SN; TD; TG

- end of record -

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Display 5/3/17 (Item 8 from file: 399)

DIALOG(R)File 399:CA SEARCH(R)

(c) 2004 American Chemical Society. All rts. reserv.

128229359 CA: 128(19)229359h PATENT

Immunoglobulin trans-spliced transcripts and uses thereof

INVENTOR(AUTHOR): Saxon, Andrew W.; Ke, Zhang; Fujieda, Shigeharu

LOCATION: USA

ASSIGNEE: Regents of the University of California; Saxon, Andrew W.; Ke, Zhang; Fujieda, Shigeharu

PATENT: PCT International ; WO 9807738 A1 DATE: 19980226

APPLICATION: WO 97US15485 (19970815) \*US 23579 (19960819)

PAGES: 84 pp. CODEN: PIXXD2 LANGUAGE: English CLASS: C07H-021/04A; C12N-007/00B; C12N-015/11B; A61K-048/00B; C12P-019/34B

DESIGNATED COUNTRIES: AL; AM; AT; AU; AZ; BA; BB; BG; BR; BY; CA; CH; CN; CU; CZ; DE; DK; EE; ES; FI; GB; GE; HU; IL; IS; JP; KE; KG; KP; KR; KZ; LC; LK; LR; LS; LT; LU; LV; MD; MG; MK; MN; MW; MX; NO; NZ; PL; PT; RO; RU; SD; SE; SG; SI; SK; TJ; TM; TR; TT; UA; UG; UZ; VN; AM; AZ; BY; KG; KZ; MD; RU; TJ; TM DESIGNATED REGIONAL: GH; KE; LS; MW; SD; SZ; UG; ZW; AT; BE; CH; DE; DK; ES; FI; FR; GB; GR; IE; IT; LU; MC; NL; PT; SE; BF; BJ; CF; CG; CI;

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Display 5/3/17 (Item 8 from file: 399)

DIALOG(R)File 399:CA SEARCH(R)

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CM; GA; GN; ML; MR; NE; SN; TD; TG

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Display 5/3/18 (Item 1 from file: 34)

DIALOG(R)File 34:SciSearch(R) Cited Ref Sci

(c) 2004 Inst for Sci Info. All rts. reserv.

12216223 Genuine Article#: 709CG No. References: 0

Title: **Trans-splicing AAV** vector system expands the

packaging capacity of **AAV** gene therapy vectors for delivery of  
larve-transgenes to the retina  
Author(s): Reich SJ; Maguire AM; Auricchio A; Hildinger M; Wilson JM;  
Bennett J  
Corporate Source: Univ Penn, Scheie Eye Inst, Dept  
Ophthalmol, Philadelphia//PA/19104; Univ Penn, Inst Human Gene  
Therapy, Philadelphia//PA/19104  
Journal: INVESTIGATIVE OPHTHALMOLOGY & VISUAL SCIENCE, 2002, V43, 2 (MAY)  
, PU1305-U1305  
ISSN: 0146-0404 Publication date: 20020500  
Publisher: ASSOC RESEARCH VISION OPHTHALMOLOGY INC, 12300 TWINBROOK  
PARKWAY, ROCKVILLE, MD 20852-1606 USA  
Language: English Document Type: MEETING ABSTRACT

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Display 5/3/19 (Item 2 from file: 34)  
DIALOG(R)File 34:SciSearch(R) Cited Ref Sci  
(c) 2004 Inst for Sci Info. All rts. reserv.

12051406 Genuine Article#: 676FH No. References: 0  
Title: Gene expression and molecular analysis of a **trans-  
splicing AAV** vector system In vivo and In vitro  
Author(s): Sandalon Z; Lippa MS; Lustig KH; Lock MW; Godwin SG; Lynch CM;  
Peluso RW; Burstein H  
Corporate Source: Genzyme Corp, Gene Therapy Dept, Framingham//MA/01701  
Journal: MOLECULAR THERAPY, 2003, V7, N5, 2 (MAY), PS350-S351  
ISSN: 1525-0016 Publication date: 20030500  
Publisher: ACADEMIC PRESS INC ELSEVIER SCIENCE, 525 B ST, STE 1900, SAN  
DIEGO, CA 92101-4495 USA  
Language: English Document Type: MEETING ABSTRACT

- end of record -

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Display 5/3/20 (Item 1 from file: 73)  
DIALOG(R)File 73:EMBASE  
(c) 2004 Elsevier Science B.V. All rts. reserv.

12500468 EMBASE No: 2004091905  
Current Status of Gene Therapy for Inherited Lung Diseases  
Driskell R.A.; Engelhardt J.F.  
R.A. Driskell, Ctr. Gene Ther. Cystic Fibrosis O., Dept. of Anatomy and  
Cell Biology, University of Iowa, Iowa City, IA 52242 United States  
Annual Review of Physiology ( ANNU. REV. PHYSIOL. ) (United States)  
2003, 65/- (585-612)  
CODEN: ARPHA ISSN: 0066-4278  
DOCUMENT TYPE: Journal ; Review  
LANGUAGE: ENGLISH SUMMARY LANGUAGE: ENGLISH  
NUMBER OF REFERENCES: 143

- end of record -

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Display 5/3/21 (Item 2 from file: 73)  
DIALOG(R)File 73:EMBASE  
(c) 2004 Elsevier Science B.V. All rts. reserv.

12271791 EMBASE No: 2003385611  
Gene therapy in 2003: Breadth and vigor  
Kohn D.B.  
Molecular Therapy ( MOL. THER. ) (United States) 01 SEP 2003, 8/3 (351)  
CODEN: MTOHC ISSN: 1525-0016  
DOCUMENT TYPE: Journal ; Editorial  
LANGUAGE: ENGLISH

- end of record -

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Display 5/3/22 (Item 3 from file: 73)  
DIALOG(R)File 73:EMBASE  
(c) 2004 Elsevier Science B.V. All rts. reserv.

11814786 EMBASE No: 2002387021  
Gene therapy progress and prospects: Cystic fibrosis  
Griesenbach U.; Ferrari S.; Geddes D.M.; Alton E.W.F.W.  
U. Griesenbach, Department of Gene Therapy, Faculty of Medicine, National  
Heart and Lung Institute, London SW3 6LR United Kingdom  
Gene Therapy ( GENE THER. ) (United Kingdom) 2002, 9/20 (1344-1350)  
CODEN: GETHE ISSN: 0969-7128  
DOCUMENT TYPE: Journal ; Review  
LANGUAGE: ENGLISH SUMMARY LANGUAGE: ENGLISH  
NUMBER OF REFERENCES: 65

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Display 5/3/23 (Item 1 from file: 266)  
DIALOG(R)File 266:FEDRIP  
Comp & dist by NTIS, Intl Copyright All Rights Res. All rts. reserv.

00354796  
IDENTIFYING NO.: 1R43HL72687-01 AGENCY CODE: CRISP  
Repair of Factor VIII by targeted RNA **trans splicing**  
PRINCIPAL INVESTIGATOR: MANSFIELD, S G  
ADDRESS: SGM@INTRONN.NET INTRONN, INC SOUTH BUILDING, SUITE 210  
PERFORMING ORG.: INTRONN, LLC, ROCKVILLE, MARYLAND  
SPONSORING ORG.: NATIONAL HEART, LUNG, AND BLOOD INSTITUTE  
DATES: 2004/15/03 TO 2004/14/04 FY : 2003

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Display 5/3/24 (Item 2 from file: 266)  
DIALOG(R)File 266:FEDRIP  
Comp & dist by NTIS, Intl Copyright All Rights Res. All rts. reserv.

00353434  
IDENTIFYING NO.: 7R01HL68215-02 AGENCY CODE: CRISP  
Gene Therapy for the Hemophilias  
PRINCIPAL INVESTIGATOR: WALSH, CHRISTOPHER E.  
ADDRESS: CHRISTOPHER.E.WALSH@MSSM.EDU MOUNT SINAI SCHOOL OF MEDICINE  
PERFORMING ORG.: MOUNT SINAI SCHOOL OF MEDICINE OF NYU, NEW YORK, NEW  
YORK  
SPONSORING ORG.: NATIONAL HEART, LUNG, AND BLOOD INSTITUTE  
DATES: 2007/01/02 TO 2010/31/07 FY : 2002

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Display 5/3/25 (Item 3 from file: 266)  
DIALOG(R)File 266:FEDRIP  
Comp & dist by NTIS, Intl Copyright All Rights Res. All rts. reserv.

00312566  
IDENTIFYING NO.: 1R01AR49419-01A1 AGENCY CODE: CRISP  
Dual **AAV** Vectors for Duchenne Muscular Dystrophy Therapy  
PRINCIPAL INVESTIGATOR: DUAN, DONGSHENG  
ADDRESS: DUAND@HEALTH.MISSOURI.EDU UNIV OF MISSOURI-COLUMBIA M616 MED  
SCIS BLDG  
PERFORMING ORG.: UNIVERSITY OF MISSOURI COLUMBIA, COLUMBIA, MISSOURI  
SPONSORING ORG.: NATIONAL INSTITUTE OF ARTHRITIS AND MUSCULOSKELETAL AND

SKIN DISEASES

DATES: 2007/14/03 TO 2004/30/08 FY : 2003

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Display 5/3/26 (Item 1 from file: 315)

DIALOG(R)File 315:ChemEng & Biotec Abs

(c) 2004 DECHEMA. All rts. reserv.

488669 CEABA Accession No.: 33-04-005285 DOCUMENT TYPE: Journal

Title: Stretching it: expanded applications of recombinant **adeno-**

\*\*\*associated\*\*\* viral vectors for gene therapy.

AUTHOR: Dongsheng, D. ; Engelhardt, J.F.

CORPORATE SOURCE: Univ. of Iowa College of Medicine, Iowa City, IA, USA

JOURNAL: Biotech News Int., Volume: 6, Issue: 2, Page(s): 10-12

AVAILABILITY: <http://www.gmed.net>

PUBLICATION DATE: 2001 (20010000)

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Display 5/3/27 (Item 1 from file: 357)

DIALOG(R)File 357:Derwent Biotech Res.

(c) 2004 Thomson Derwent & ISI. All rts. reserv.

0323022 DBR Accession No.: 2003-24162

Double strand interaction is the predominant pathway for intermolecular

recombination of **adeno-associated** viral genomes - for use

in gene therapy

AUTHOR: YUE YP; DUAN DS

CORPORATE AFFILIATE: Univ Missouri Univ Missouri Univ Iowa

CORPORATE SOURCE: Duan DS, Univ Missouri, Sch Med, Dept Mol Microbiol and Immunol, 1 Hosp Dr, Room M610G, MSB, Columbia, MO 65212 USA

JOURNAL: VIROLOGY (313, 1, 1-7) 2003

ISSN: 0042-6822

LANGUAGE: English

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Display 5/3/28 (Item 2 from file: 357)

DIALOG(R)File 357:Derwent Biotech Res.

(c) 2004 Thomson Derwent & ISI. All rts. reserv.

0312949 DBR Accession No.: 2003-14089

Barriers to and new approaches for gene therapy and gene delivery in cystic

fibrosis - limitations to gene transfer efficiency with application in

cystic fibrosis gene therapy; a review

AUTHOR: FERRARI S; GEDDES DM; ALTON EFWF

CORPORATE AFFILIATE: Univ London Imperial Coll Sci Technol and Med

CORPORATE SOURCE: Ferrari S, Univ London Imperial Coll Sci Technol and Med,

Dept Gene Therapy, Natl Heart and Lung Inst, Fac Med, UK Cyst Fibrosis

Gene Therapy Consortium, London SW3 6LR, England

JOURNAL: ADVANCED DRUG DELIVERY REVIEWS (54, 11, 1373-1393) 2002

ISSN: 0169-409X

LANGUAGE: English

- end of record -

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Display 5/3/29 (Item 3 from file: 357)

DIALOG(R)File 357:Derwent Biotech Res.

(c) 2004 Thomson Derwent & ISI. All rts. reserv.

0308079 DBR Accession No.: 2003-09864

Consequences of DNA-dependent protein kinase catalytic subunit deficiency

on recombinant **adeno-associated** virus genome  
circularization and heterodimerization in muscle tissue - **adeno-associated** virus vector-mediated gene transfer and expression in  
host cell for gene therapy

AUTHOR: DUAN DS; YUE YP; ENGELHARDT JF

CORPORATE AFFILIATE: Univ Missouri Univ Missouri Univ Iowa Univ Iowa  
Univ Iowa

CORPORATE SOURCE: Duan DS, Univ Missouri, Sch Med, Dept Mol Microbiol and  
Immunol, 1 Hosp Dr, Room M610G, MSB, Columbia, MO 65212 USA

JOURNAL: JOURNAL OF VIROLOGY (77, 8, 4751-4759) 2003

ISSN: 0022-538X

LANGUAGE: English

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Display 5/3/30 (Item 4 from file: 357)

DIALOG(R)File 357:Derwent Biotech Res.

(c) 2004 Thomson Derwent & ISI. All rts. reserv.

0301209 DBR Accession No.: 2003-02994

Efficient **trans-splicing** in the retina expands the utility of  
**adeno-associated** virus as a vector for gene therapy -  
recombinant **adeno-associated** virus vector-mediated gene  
transfer, expression in host cell useful for gene therapy

AUTHOR: REICH SJ; AURICCHIO A; HILDINGER M; GLOVER E; MAGUIRE AM;  
WILSON JM; BENNETT J

CORPORATE AFFILIATE: Scheie Eye Inst Univ Penn Univ Penn Telethon Inst  
Genet and Med

CORPORATE SOURCE: Bennett J, Scheie Eye Inst, Dept Ophthalmol, FM Kirby Ctr  
Mol Ophthalmol, Stellar Chance Labs 310, 422 Curie Blvd, Philadelphia,  
PA 19104 USA

JOURNAL: HUMAN GENE THERAPY (14, 1, 37-44) 2003

ISSN: 1043-0342

LANGUAGE: English

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Display 5/3/31 (Item 5 from file: 357)

DIALOG(R)File 357:Derwent Biotech Res.

(c) 2004 Thomson Derwent & ISI. All rts. reserv.

0296320 DBR Accession No.: 2002-18167 PATENT

Preparing target cell-specific pharmaceutical, useful for gene therapy of  
e.g. cancer, by cloning expression control sequences having higher  
activity in target cell - recombinant adeno virus vector plasmid  
pAdEasy-1-mediated gene transfer and expression in HEK-293 for use in  
gene therapy

PATENT ASSIGNEE: DOOLEY S 2002

PATENT NUMBER: DE 10057014 PATENT DATE: 20020529 WPI ACCESSION NO.:  
2002-509915 (200255)

PRIORITY APPLIC. NO.: DE 1057014 APPLIC. DATE: 20001117

NATIONAL APPLIC. NO.: DE 1057014 APPLIC. DATE: 20001117

LANGUAGE: DE

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Ref	Items	Index-term
E1	1	AU=ENGELHARDT, JOERG
E2	10	AU=ENGELHARDT, JOHANN
E3	12	*AU=ENGELHARDT, JOHN
E4	1	AU=ENGELHARDT, JOHN A.



E5	21	AU=ENGELHARDT, JOHN F
E6	116	AU=ENGELHARDT, JOHN F.
E7	3	AU=ENGELHARDT, JOHN FREDERIC
E8	9	AU=ENGELHARDT, JOHN J.
E9	2	AU=ENGELHARDT, JOHN JOSEPH
E10	2	AU=ENGELHARDT, JOHN K.
E11	2	AU=ENGELHARDT, JOHN KERCH
E12	6	AU=ENGELHARDT, JOHN S.

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Ref	Items	Index-term
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E4	175	AU=ENGELHARDT JOHN F
E5	1	AU=ENGELHARDT JOHN JOSEPH
E6	6	AU=ENGELHARDT JOHN K
E7	2	AU=ENGELHARDT JOSEF I
E8	2	AU=ENGELHARDT JOSEPH
E9	1	AU=ENGELHARDT JOSEPH B
E10	6	AU=ENGELHARDT JOSEPH I
E11	1	AU=ENGELHARDT JOZEF I
E12	1	AU=ENGELHARDT JOZSEF

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E3	44	*AU=DUAN, DONGSHENG
E4	4	AU=DUAN, DONGYONG
E5	1	AU=DUAN, DONGYUAN
E6	1	AU=DUAN, DUOLU
E7	1	AU=DUAN, E
E8	6	AU=DUAN, E.
E9	1	AU=DUAN, E. K
E10	15	AU=DUAN, E. K.
E11	6	AU=DUAN, EN-KUI
E12	30	AU=DUAN, ENKUI

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? e au=duan dongsheng

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E3	37	*AU=DUAN DONGSHENG
E4	1	AU=DUAN DP
E5	12	AU=DUAN DR
E6	42	AU=DUAN DS
E7	5	AU=DUAN DSR
E8	2	AU=DUAN DUAN LIU
E9	1	AU=DUAN DUAN XUEFEI
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E11	3	AU=DUAN DX
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